**Forfa, Tracey H**
**From:** Scott R. Burger [celltherapy@earthlink.net]
**Sent:** Tuesday, June 25, 2002 3:01 AM
**To:** TForfa@OC.FDA.GOV
**Subject:** Slides from ISCT presentation at FDA hearing

Tracey Forfa, Esq.
Office of the Ombudsman

Dear Ms. Forfa,

Here are the slides for the presentation Steve Noga and I, representing the International Society for Cellular Therapy, gave at the FDA hearing on combination products. I hope these will be helpful to you.

Please let me know if you have any questions or would like to discuss this further. I have included contact information for both Steve Noga and myself in this email, below, as well as in the last slide of the attached file. I would be pleased to be of help to you.

Best regards,

Scott

Scott R. Burger, MD

Advanced Cell & Gene Therapy Consulting

105 Highgrove Drive

Chapel Hill, NC 27516

919-933-3482 – voice/fax

919-414-6947 – mobile

celltherapy@earthlink.net

(Executive Board member, Editor

International Society for Cellular Therapy)

Stephen J. Noga, MD, PhD
Director, Hematology/Medical Oncology
Director, BMT & Cellular Therapeutics Program
Alvin & Lois Lapidus Cancer Institute
Sinai Hospital of Baltimore
2401 West Belvedere Avenue
Baltimore, MD 21215
410-601-4710
410-601-8448 – Fax
snoga@lifebridgehealth.org

FDA Part 15 Hearing - 06-24-20

(President, International Society for Cellular Therapy)

02N-0169

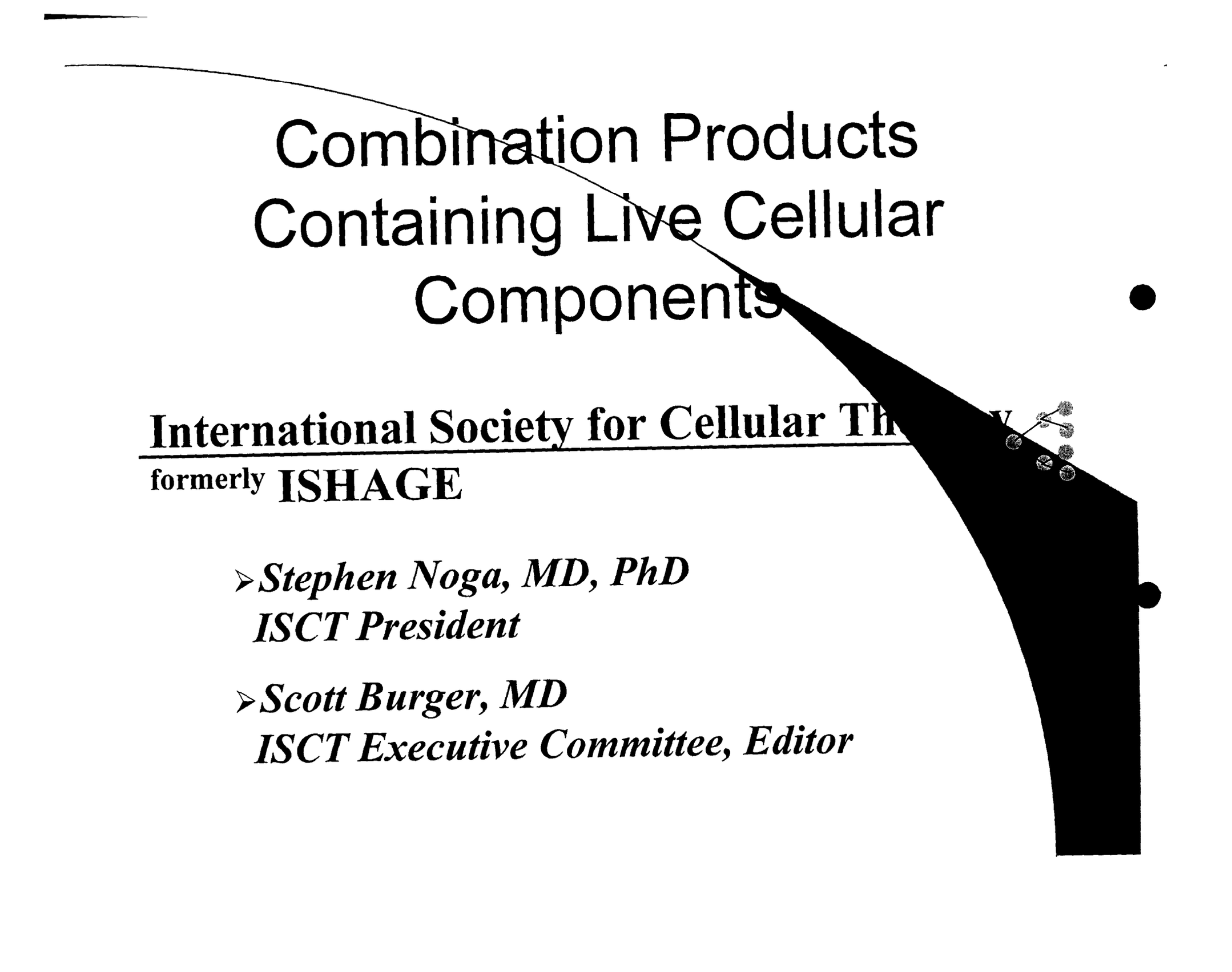
TS3

# Combination Products Containing Live Cellular Components

**International Society for Cellular Th[illegible]y**

formerly **ISHAGE**

- ***Stephen Noga, MD, PhD***
  ***ISCT President***
- ***Scott Burger, MD***
  ***ISCT Executive Committee, Editor***



# International Society for Cellular Therapy

formerly **ISHAGE**

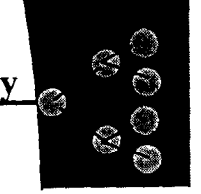
- Represents scientists, technologists and regulatory individuals within the evolving field of cellular therapy. This includes all aspects of
  - Cellular therapy
  - Tissue-based therapy
  - Genetic manipulation and gene therapy
- Share common interests with AABB, ASBM and other closely allied societies

# Implications of Regulatory Structure

- **Although focus of today's hearing is on combination products for wound hea ng, eventual regulatory structure chosen will have b der effects**
- **Range of similarly defined combination prod currently being developed, involving diverse t of cells and tissues**
  - **Encapsulated pancreatic islet cells**
  - **Hepatocyte-based liver assist devices**
  - **Mesenchymal cell-based structural grafts**

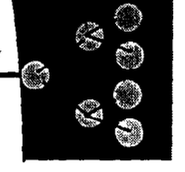
# Combination Products - Living Cells with Non-Living Matrix

- **Appropriate, reasonable regulatory oversight needed for both elements of combination product**
- **Determine regulatory structure based on element most in need of control**
  - **Which element is the most critical, complex, variable?**
  - **Which element most affects long term outcome?**
  - **Which element is most affected by environment?**
- **Becomes more complex if genetically-modified cells *and/or* matrix used in future protocols**



# Non-Living Matrix

- **May be complex in nature, but basic safety profile more readily established**
  - **Safety, purity, potency testing more likely use established analytical methods**
  - **Larger *(consistency)* lot sizes**
  - **Amenable to biochemical characterization**



# Living Cellular Element

- **Can be expected to be more comple[illegible] difficult to control**
  - **Safety, purity, potency more difficult to evalu[illegible]**
  - **May require development, validation of novel a[illegible] methods**
  - **Manufacture may involve extensive, complex cell a[illegible] tissue engineering, gene modification**
  - **Limited availability of test samples – lot size *(may be unique to each patient in some applications)***

# Basis For Regulatory Approach

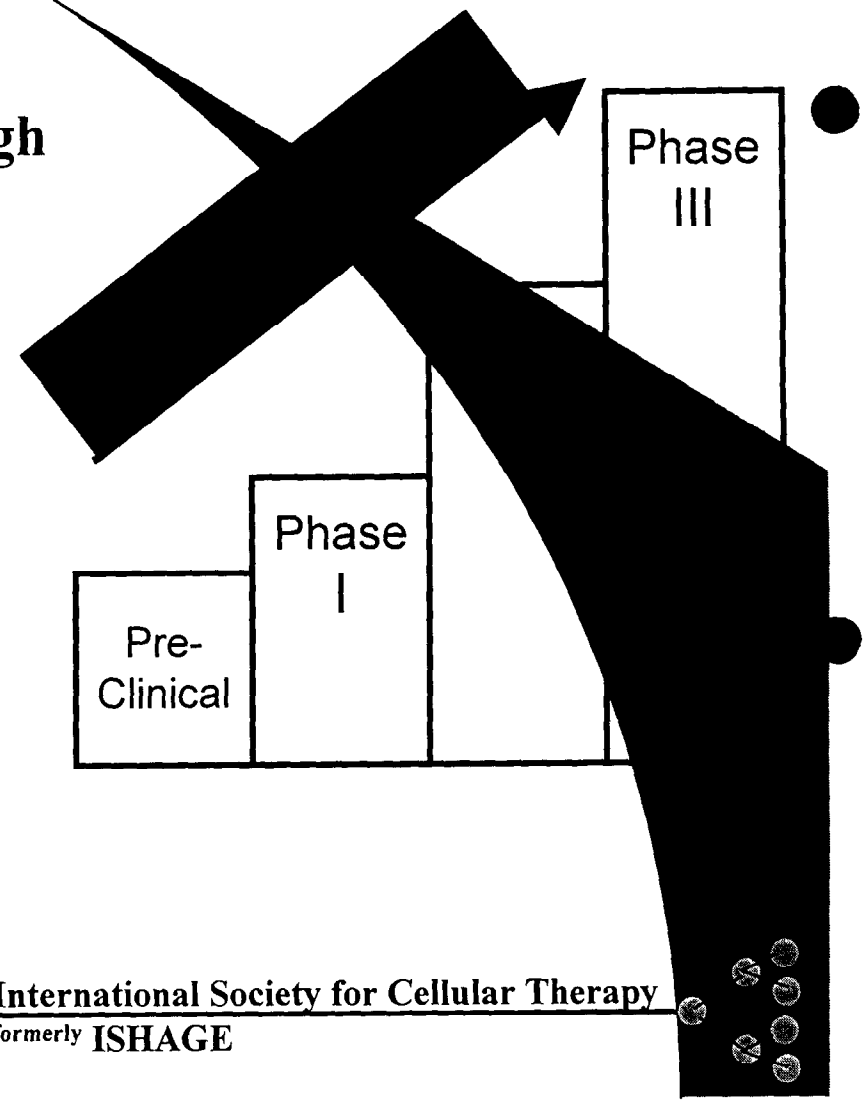
- **Living cellular element therefore most [illegible]itical**
- **Cells, Tissues & Gene therapies currently [illegible]ted by CBER:**
  - **Experience in biological products**
  - **Experience in unusual issues specific to cellular ther[illegible]**
  - **Early experience with hematopoietic cell/matrix devic[illegible]**
  - **Established relationship with many experts in the field**

# Specific Recommendations I

- **CBER serve as the primary agency for these combination products**
- **Full use of CDRH expertise must be assured**
  - **Example - Productive and valuable cooperation between CBER and CDER**
- **Two product reviewers for IND applications combination products**
  - **CBER – provide expertise in biological products**
  - **CDRH – provide expertise in non-living support matrices**
- **Representation of CDRH within the new CBER office of Cells, Tissues & Gene Therapy**

# Specific Recommendations II

- **Continue stepwise approach used in regulation of cell therapies**
  - ➢ **↑ Control, characterization through product development**
- **Appropriate levels of control for initial academic-based trials of novel therapies in life-threatening diseases**
  - ➢ **Priority of safety, but avoid impeding trials altogether by imposing Phase III-level requirements in early trials**



Stephen J. Noga, MD, PhD -- President, International Society for Cell
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